CLINICAL TRIALS IN DOWN SYNDROME FOR CO-OCCURRING CONDITIONS ACROSS THE LIFESPAN WORKSHOP

AGENDA

Day 1 – May 7, 2020

8:30 a.m. NIH Welcome
Diana W. Bianchi
Eunice Kennedy Shriver National Institute of Child Health and Human Development (NICHD)

Introduction of Workshop
Laurie Ryan, National Institute on Aging (NIA)

Overview of Workshop Logistics
Erika Tarver, NIA

8:40 a.m. Prenatal Treatment: Clinical Trial of the Future?
Diana W. Bianchi, NICHD

9:00 a.m. Session: Pediatric/Developmental Conditions
(co-chairs: Priya Kishnani and Steve Abman)

9:05 a.m.
• Pediatric Cardiology Consideration in Children with Down Syndrome
  Ashraf Harahsheh, Children’s National Health System

9:20 a.m.
• Sleep conditions
  o Role of Sleep for Cognitive Development in Down Syndrome
    Jamie Edgin, University of Arizona

9:35 a.m.
  o Sleep Apnea in Children with Down Syndrome
    Daniel Combs, University of Arizona

9:50 a.m.
• Take a Breath: Post-Influenza-Like State in Down Syndrome
  Michael Yeager, University of Colorado Denver

10:05 a.m.
• Infectious Diseases in People with Down Syndrome
  Andrew Nowalk, University of Pittsburgh

10:25 a.m.
• JAK Inhibition in Down Syndrome: Treating Autoimmunity and Beyond
  Joaquin Espinosa, University of Colorado Denver

10:45 a.m. BREAK
11:00 a.m.  
- Leukemia in Children with Down Syndrome: Treading Carefully with Clinical Trials  
  Jeffrey Taub, Wayne State University

11:15 a.m.  
- Central Nervous System Conditions  
  o Down Syndrome & ADHD  
    Anna Esbensen, Cincinnati Children’s Hospital

11:30 a.m.  
- Cognitive Considerations for Clinical Trials in Children with Down Syndrome  
  Laura Hahn, University of Illinois at Urbana-Champaign

11:45 a.m. – 12:15 p.m.  
LUNCH

12:15 p.m.  
- Communication and Language Intervention  
  o Speech, language, articulation  
    Ann P. Kaiser, Vanderbilt University

12:30 p.m.  
- Factors Affecting Listening and Speech Perception in Children with Down syndrome  
  Lori Leibold, Boys Town National Research Hospital

12:45 p.m.  
- Eye Tracking Technologies to Characterize Visual Attending in Down Syndrome: Implications for Optimizing the Design of Visual Supports for Communication  
  Krista Wilkinson, Pennsylvania State University

1:00 p.m. – 1:25 p.m.  
Session Q&A

1:25 p.m.  
Overview of Clinical Trials in the Down Syndrome Population  
Priya Kishnani, Duke University

1:45 p.m. – 3:20 p.m.  
Panel: Considerations for Participation in Clinical Trials – Pediatric and Adult Populations  
Moderator: Priya Kishnani  
[5-minute presentation per panelist + Q&A]

Discussants:
- Frank Stephens (self-advocate)  
- Ben Handen (psychologist)  
- Emily Chesnut (parent/advocate)  
- Marilyn Bull (physician)  
- Michelle Sie Whitten (parent/advocate)  
- Florence Lai (physician)  
- David Egan (self-advocate)  
- Kathleen Egan (parent/advocate)  
- Hampus Hillerstrom (parent/advocate)  
- Donna and Wayne Leigh (parent/advocate)  
- Brian Skotko (physician/sibling)
3:20 p.m.  **BREAK**

**Session: Non-pharmacological/Lifestyle Interventions**
**Session Moderator: Annie Cohen**

3:30 p.m.  • Lifestyle and Alzheimer’s Disease in Down Syndrome: Physical Activity and Cognitive Stimulation  
Sigan Hartley, University of Wisconsin – Madison

3:45 p.m.  • Music and Social Engagement: Research and Future Directions for Individuals with Down Syndrome  
Miriam Lense, Vanderbilt University

4:00 p.m.  Session Q&A

**Session: Adult/Aging Conditions**  
(co-chairs: Annie Cohen and Mike Rafii)

4:15 p.m.  • Challenges to Defining Obesity in Down Syndrome  
Andrea Kelly, Children’s Hospital of Philadelphia

4:30 p.m.  • Central Nervous System Conditions  
  ○ Alzheimer’s Disease, and other Central Nervous System Disorders  
    Michael Rafii, University of Southern California

4:45 p.m.  ○ Regression: A Review of Available Literature and Research Gaps  
    Michelle Palumbo, Massachusetts General Hospital

5:00 p.m.  Session Q&A

5:20 p.m.  **Adjourn Day 1 of Workshop**
Day 2, May 8
9:00 a.m.  
**Session: Overview of INCLUDE Project funded grants on Clinical Trials (R61/R33) and Clinical Trial Readiness (R21)**

Session Moderator: Laurie Ryan

**R21 – Clinical Trial Readiness**

9:05 a.m.  
- Epigenetic Silencing of Trisomy HSA21  
  Volney Sheen, Beth Israel Deaconess

9:10 a.m.  
- Using fNIRS to Study the Neural Correlates of Executive Function and Sleep Impairment in Down Syndrome  
  Nancy Raitano Lee, Drexel University

9:15 a.m.  
- Acceptability and Performance on In-Home Polysomnography in Youth with Down Syndrome  
  Ignacio Tapia, Children’s Hospital of Philadelphia

9:20 a.m.  
- Measures of Pulmonary Health in Children with Down Syndrome  
  Emily DeBoer, University of Colorado Denver

9:25 a.m.  
- Early Risk for ADHD Symptoms in Young Children with Down Syndrome  
  Deborah Fidler, Colorado State University

9:30 a.m.  
- Bronchus-Associated Lymphoid Tissue: Friend or Foe in Down Syndrome  
  Michael Yeager, University of Colorado Denver

9:35 a.m.  
- Down Syndrome, Early Cataracts, Eye Diseases, and Beta-amyloid Conformers  
  Geoffrey Chang, University of California, San Diego

**R61/R33 – Clinical Trials Development**

9:40 a.m.  
- Medications for Obstructive Sleep Apnea to Improve Cognition in Children with Down Syndrome (MOSAIC DS)  
  Daniel Combs, University of Arizona
9:45 a.m.  • Evaluating Assessment and Medication Treatment of ADHD in Children with Down Syndrome
Anna Esbensen, Cincinnati Children’s Hospital

9:50 a.m.  • JAK Inhibition in Down Syndrome
Joaquín Espinosa, University of Colorado Denver

9:55 a.m.  • Clinical trials to Prevent Alzheimer’s Disease in Down Syndrome: Trial Ready Cohort – Down syndrome (TRC-DS)
Michael Rafii, University of Southern California

10:00 a.m.  • Positive Airway Pressure for the Treatment of the Obstructive Sleep Apnea Syndrome in Children with Down Syndrome
Ignacio Tapia, Children’s Hospital of Philadelphia

10:15 a.m.  BREAK

10:30 a.m.  Session: Clinical Trial Infrastructure
Session Moderator: Steve Abman
10:30 a.m.  • Down Syndrome Patient Registries
Steve Abman, University of Colorado Denver

10:45 a.m.  • DS-Connect®
Sujata Bardhan, NICHD

11:00 a.m.  • PTN-DS: Clinical Pharmacokinetics and Safety Trials in Down Syndrome
Mara Becker, Duke University

11:15 a.m.  • Pediatric Heart Network
Julie Miller, HealthCore

11:30 a.m.  • Alzheimer’s Clinical Trial Consortium – Down Syndrome (ACTC-DS)
Michael Rafii, University of Southern California

11:45 a.m.  Session Q&A

12:00 p.m. – 12:30 p.m.  LUNCH
12:30 p.m.  **Session: Recruitment into Clinical Trials**  
Session Moderator: Mike Rafii

- Recruitment of Diverse Populations, Lessons Learned and Implications for Down Syndrome Research  
  Annie Cohen, University of Pittsburgh

- Consent
  - Obtaining Consent into Clinical Trials: State Specific Policies and Requirements  
    Sharon Krinsky-McHale, New York State Institute for Basic Research in Developmental Disabilities

12:45 p.m.

1:00 p.m.

- Obtaining Consent or Assent  
  Ben Handen, University of Pittsburgh

1:15 p.m.  Sessions Q & A

1:30 p.m.  **Report from Break-out Teleconference Sessions**

- Pediatric Issues and Considerations  
  Steve Abman and Priya Kishnani

- Adult Issues and Considerations  
  Annie Cohen and Mike Rafii

2:10 p.m.  Breakout Session Reports Q & A

2:30 p.m.  **Closing Remarks**  
Gary H. Gibbons  
National Heart, Lung and Blood Institute

2:45 p.m.  **Adjourn Workshop**